
Disease research and drug development models based on genetically altered human embryonic stem cells.

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Public Summary:

Scientific Abstract:

Mouse models have become an indispensable tool to study the pathogenesis of human diseases. However, due to the apparent cellular and physiological differences between mouse and human, mouse models often fail to faithfully recapitulate certain defects observed in human patients. In addition, these differences contribute to the dilemma in drug development that many therapeutic strategies work well in mouse models but do poorly in human patients. Therefore, it has become increasingly important to develop additional physiologically relevant human disease models for mechanistic studies and drug development. With the unlimited self-renewal capability and the pluripotency to differentiate into all cell types in the body, human embryonic stem cells (hESCs) with causative genetic mutations as well as their differentiated derivatives represent the much needed human disease models for studies on disease mechanisms and for drug development. Here we summarize recent progresses in developing hESCs into human disease models.

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